

CLAIMS

1. Use of a nucleic acid of interest for the manufacture of a composition for intravenous administration to a human, for the treatment of a subject affected by or susceptible to being affected by a CNS disorder, wherein said composition is a composition enriched in human cells expressing the CD34 marker or human cells capable of giving rise to cells expressing the CD34 marker, at least a portion of said cells comprising a nucleic acid of interest, and wherein at least a portion of said administered cells are capable of migrating to the CNS and expressing the nucleic acid of interest in the CNS of this subject.

2. Use according to claim 1, wherein said administered cells are capable of giving rise to microglia in the CNS of a mammal.

3. Use of a nucleic acid sequence encoding a polypeptide of interest for the manufacture of a composition for intravenous administration to a human, for the treatment of a subject affected by or susceptible to being affected by a CNS disorder under conditions that result in the expression of a polypeptide of interest at a level that provides a therapeutic effect in said subject, wherein said composition is a composition comprising hematopoietic progenitor cells or hematopoietic stem cells isolated from cells comprising hematopoietic progenitor or hematopoietic stem cells obtained from a human subject, and wherein a nucleic acid encoding a polypeptide of interest has been introduced to said isolated hematopoietic progenitor or stem cell.

4. Use of cells for the manufacture of a composition for intravenous administration to a human, for the treatment of a subject affected by or susceptible to being affected by a CNS disorder, wherein said composition is a composition enriched in human cells expressing the CD34 marker or human cells capable of giving rise to cells expressing the CD34 marker, and wherein at least a portion of said administered cells are capable of migrating to the CNS and giving rise to microglia.

5. Use according to one of claims 1 to 4, wherein said administration results in a reduction in the severity of central nervous system damage or symptoms of a central nervous system disorder.

6. Use of a nucleic acid of interest for the manufacture of a composition for intravenous administration to a human, for the treatment of a subject affected by or

susceptible to being affected by a CNS disorder under conditions that result in the expression of a polypeptide of interest at a level that provides a therapeutic effect in said subject, wherein said composition is a composition enriched in human cells expressing the CD34 marker or human cells capable of giving rise to cells expressing the CD34 marker, at least of portion of said cells being recombinant cells comprising a nucleotide sequence encoding said polypeptide operably linked to expression control elements.

7. Use according to claim 6, wherein at least a portion of said administered cells migrate to the CNS, give rise to microglia and express the nucleic acid of interest in the CNS of said subject.

10 8. Use according to one of claims 1 to 7, wherein said administered cells expressing the CD34 marker, said cells capable of giving rise to cells expressing the CD34 marker, or said hematopoietic progenitor or hematopoietic stem cells differentiate into a microglia cell.

9. Use according to one of claims 3 and 6, wherein at least a portion of said administered cells express the nucleic acid of interest in the CNS of said subject.

10. Use according to one of claims 1, 4 and 6, wherein at least 20 % of cells in said cell composition express the CD34+ marker.

11. Use according to one of claims 1, 4 and 6, wherein the subject to be treated is pretreated in order to enhance engraftment of said cells expressing the CD34 marker, cells capable of giving rise to cells expressing the CD34 marker, hematopoietic progenitor or stem cells.

12. Use according to one of claims 1, 4 and 6, wherein said cells expressing the CD34+ marker, cells capable of giving rise to cells expressing the CD34 marker, hematopoietic progenitor cells or hematopoietic stem cells are prior isolated.

25 13. Use according to claim 4, wherein said cells expressing the CD34 marker or cells capable of giving rise to cells expressing the CD34 marker are recombinant cells comprising a nucleic acid of interest.

14. Use according to one of claims 1, 3, 4 and 6, wherein at least a portion of said cells expressing the CD34+ marker, cells capable of giving rise to cells expressing the CD34 marker, hematopoietic progenitor cells or hematopoietic stem cells are transduced with a vector comprising a nucleic acid of interest operably linked to a promotor capable of effecting the expression of said nucleic acid of interest in said cells.

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15. Use according to one of claims 1, 3, 4 and 6, wherein at least a portion of said cells expressing the CD34+ marker, cells capable of giving rise to cells expressing the CD34 marker, hematopoietic progenitor cells or hematopoietic stem cells are transduced with a viral vector, preferably with a lentiviral vector.

5 16. Use according to claim 3, wherein said hematopoietic progenitor or hematopoietic stem cells express the CD34+ marker or are capable of differentiating into cells expressing the CD34+ marker.

17. Use according to one of claims 1, 4 and 6, wherein said cells expressing the CD34+ marker or cells capable of giving rise to cells expressing the CD34 marker
10 are hematopoietic progenitor cells or hematopoietic stem cells.

18. Use according to one of claims 1, 3, 6 and 13, wherein said nucleic acid encodes a non-secreted or a secreted protein.

19. Use according to one of claims 1 to 18, wherein the CNS disorder which affects or which is susceptible to affect the subject is characterized by diffuse
15 neurodegeneration, preferably the Alzheimer's disease.

20. Use according to one of claims 1 to 19, wherein the administered cells are autologous to the subject to be treated.

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